SPECIFICATION

Methods for design and selection of short double-stranded oligonucleotides, and compounds of gene drugs

ABSTRACT

The present invention provides methods for designing and selecting efficacious SDSOs as a gene drug that can specifically inactivate a group of corresponding genes. In particular, this invention relates to a process including the recruitment of target genes causing a disease, the identification of an endogenous siRNA sequence, the prediction of an efficacious SDSO, and the assembly of one or more SDSOs into related carriers with the ability targeting to diseased a cell or a tissue. This invention further includes pharmaceutical compounds of a gene drug, particularly one or more 21nt double-stranded oligonucleotides with a 5'-AU(T)CCG -3' or 5'-U(T)CCCG -3' cleavage pattern in its antisense strand, which can specifically hybridize with a 5'-CGGAU(T)-3' or 5'-CGGGA-3' motif in a or more cognate RNA molecules such as a primary transcript or an mRNA. Methods of using these compounds for treatment of diseases or disorders associated with expression of one or a group of genes in a cell or tissue of the human or other animals are also provided.

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